Phase 3 Clinical Trial Targeting Lou Gehrig's Disease Gets $15.9 Million Investment From Stem Cell Agency

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July 20, 2017 Oakland, CA Amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, is a particularly nasty, almost always fatal neurological condition that has no effective treatment and no cure. Now the California Institute for Regenerative Medicine (CIRM) is investing $15.9 million in a therapy to try and change that.

ALS attacks motor neurons, the cells in the brain and spinal cord that control our muscles and movement. Over time the disease destroys a person's ability to speak, to swallow, to move, even to breathe.

BrainStorm Cell Therapeutics has developed a therapy they hope will slow down, and potentially even stop, the progression of the disease.

"ALS is a devastating disease with an average life expectancy of less than five years and individuals afflicted with this condition suffer an extreme loss in quality of life," says Maria Millan, MD, CIRM's interim President & CEO. "CIRM is partnering with BrainStorm to follow up on the company's promising Phase 2 trial in ALS. CIRM's mission is to accelerate stem cell treatments to patients with unmet medical needs and, in keeping with this mission, our objective is to find a treatment for patients ravaged by this neurologic condition for which there is currently no cure."

BrainStorm uses mesenchymal stem cells that are taken from the patient's own bone marrow. These stem cells are then modified to boost their production of neurotrophic factors, which are known to help support and protect neurons, the cells destroyed by the disease. The CIRM funding will enable the company to test this therapy, called NurOwn, in a Phase 3 trial involving around 200 patients.

In an earlier Phase 2 clinical trial, involving 48 people with ALS, NurOwn was shown to be safe and well-tolerated. The trial also showed it could halt or reverse disease progression over six months, compared to a placebo.

"We are honored to be awarded this CIRM grant, and appreciate the support of CIRM in the development of NurOwn," said Chaim Lebovits, President and CEO of BrainStorm. "This substantial award provides further validation for our technology and clinical program, and recognizes the importance of developing effective treatments for patients afflicted with ALS."

The CIRM Board today also awarded $5.79 million to Dr. Ezra Cohen at the University of California, San Diego to develop a genetically modified therapy to attack cancer stem cells. These are cells that are believed to be able to evade standard treatments, such as chemotherapy and radiation, enabling the cancer to return and spread. The funding will enable Cohen to do the research needed to apply for clearance from the Food and Drug Administration for a clinical trial.

Cohen is using what are called specific chimeric antigen receptor T cells, or CAR T cells. The cells are taken from the patient's own blood, then modified so they specifically target cancer stem cells that express a protein called ROR1 on their surface. It's hoped that after being transfused back into the patient, the CAR T cells will then recognize and kill the cancer stem cells that have ROR1 markers. And because these CAR T cells can remain in the body long after they have been infused, it's hoped they will prevent cancer recurrence and result in long-term remission.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution

dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to **www.cirm.ca.gov**
